WASHINGTON STATE Board of Health
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Washington State Department of Health

NEWBORN SCREENING PROGRAM
CYSTIC FIBROSIS ADVISORY COMMITTEES FINDINGS
Report to the Board – October 12, 2005

The following is a summary of the findings of two advisory committees that were convened to review newborn screening to detect cystic fibrosis (CF) under the framework of the Board's Criteria for Adding Disorders to the Newborn Screening Program (NBS). A technical committee focused specifically on medical/public health perspectives. That committee determined that broader review was warranted, and a broadly representative committee was convened to conduct additional review.

Also, the Cystic Fibrosis Advisory Committee made additional observations and recommendations to be considered if cystic fibrosis is added to the screening panel:

- Sweat tests (which are necessary to establish the diagnosis) should occur at laboratories that have demonstrated competence and adequate volume to perform them accurately.
- To achieve the benefits of early detection, newborns identified with CF need to be assured of follow-up care though coordination with CF centers.
- CF centers will continue to practice stringent infection control measures as part of their plan to provide services to a younger population identified with CF.
- CF newborn screening outcomes should be included in on-going evaluation, and Washington State should participate in any national CF data collection efforts.

Criteria #1 - Prevention Potential and Medical rationale
Identification of the condition provides a clear benefit to the newborn: preventing delay in diagnosis; developmental impairment; serious illness or death.

CF Technical Advisory Committee Findings:
Earlier diagnosis through newborn screening can improve nutritional status and cognitive development, reduces hospital stays and 'diagnostic odysseys' and prevents some deaths.

CF Advisory Committee Findings:
Voting summary: Unanimous (14 votes); the data support conclusion that the criterion is met.
Discussion summary: The strongest data supports the notion that earlier diagnosis through newborn screening improves nutritional status.

Criteria #2 - Treatment Available
Appropriate and effective screening, diagnosis, treatment, and systems are available for evaluation and care.

CF Technical Advisory Committee Findings:
Expert treatment is essential to achieve the benefits of early diagnosis. This treatment is available at three CF specialty care centers in: Seattle, Tacoma, and Spokane.

CF Advisory Committee Findings:
Voting summary: Unanimous (14 votes); the data support conclusion that the criterion is met.
Discussion summary: Basically in agreement with CF Technical Advisory Committee.

Criteria #3 - Public Health Rationale
Nature of the condition (symptoms are usually absent, such that diagnosis is delayed and treatment effectiveness is compromised) and prevalence of the condition justify population-based screening rather than risk-based screening.

CF Technical Advisory Committee Findings:
Without newborn screening, diagnosis is typically delayed beyond a year of age, after the onset of preventable nutritional and developmental deficits. There are no reliable risk factors, so population screening is required for early diagnosis.

CF Advisory Committee Findings:
Voting summary: Unanimous (14 votes); the data support conclusion that the criterion is met.
Discussion summary: Basically in agreement with CF Technical Advisory Committee.

Criteria #4 - Available Technology
Sensitive, specific and timely tests are available that can be adapted to mass screening.

CF Technical Advisory Committee Findings:
Screening tests with suitable sensitivity and specificity are available for mass screening. The same technology is used by the department in screening for some disorders in the current mandatory screening panel.

CF Advisory Committee Findings:
Voting summary: Unanimous (14 votes); the data support conclusion that the criterion is met.
Discussion summary: A screening method that avoids DNA testing generates fewer false positives and un-necessary sweat tests, avoids identifying CF carriers (heterozygotes) and is lower cost. No screening approach, however, will detect all affected infants; about 2% will be missed. However a screening approach that does not include DNA testing may miss one additional affected child every 16 – 20 years. Provider education and a strong follow up system might be bigger challenges than testing mechanisms. Screening that avoids DNA testing appears to be a good approach, but it should be monitored over time to ensure that the benefits were accurately projected and that it is meeting expectations.

Criteria #5 - Cost-Benefit / Cost –Effectiveness
The benefits justify the costs of screening.

CF Technical Advisory Committee Findings:
No recommendation, the analysis to be completed prior to CF advisory committee meeting on July 11, 2005.

CF Advisory Committee Findings:
Voting summary: Nine votes that the analysis supports conclusion that the criterion is met; 3 votes the analysis does not support conclusion that the criterion is met; 2 abstentions
Discussion summary: Questions came up on: the use of *life years saved* as part of the *total savings* to calculate the benefit cost ratio; why improved nutritional status was not captured in the cost benefit model; why parents' costs (e.g. doctor visits and missed days of work) were not included. Finally, the assumptions made about hospitalization reductions were questioned.

Cost–Benefit Update

Since the July meeting, the Department of Health economist and Newborn Screening Program staff have carefully reviewed each of the assumptions and calculations of the cost benefit analysis. This was done in consultation and with the gracious assistance of a Senior Health Economist from the National Center on Birth Defects and Developmental Disabilities at the Centers for Disease Control and Prevention. The outcome was a revised analysis that addresses the concerns and continues to demonstrate substantial, positive benefits in relation to the costs of screening. The analysis estimates that $5.40 in benefit will be realized for each dollar spent on screening related costs.

CF Advisory Committee Members

Children - Families

1. Michael Gray, Parent
2. Cherish Hart, March of Dimes - Washington Chapter

Professional Associations

3. Invited Representative, American Academy of Pediatrics, Washington Chapter
4. Brenda Suiter, Washington State Hospital Association

Medical/Clinical Specialties

5. Margaret Rosenfeld, MD, MPH, Children's Hospital CF clinic
6. Larry Larson, DO, Mary Bridge CF clinic
7. Susan Casey, RD, CD, Children's Hospital CF clinic
8. Ian Glass, MD, Washington State Genetic Advisory Committee

Principle Payers

9. Nancy Anderson, MD, DSHS Medical Assistance Administration
10. Nancy Fisher, MD, MPH, Washington State Health Care Authority
11. Sydney Smith Zvara, Association of Washington Healthcare Plans
12. Ida Zodrow Office of the Insurance Commissioner

Medical Ethics

13. Wylie Burke, MD, PhD UW Department of Medical History and Ethics

Public Health

14. Maxine Hayes, MD, MPH, State Health Officer, Washington State Dept. of Health
15. Tom Locke, MD, MPH, Chair, Washington State Board of Health
16. Scott Lindquist, MD, MPH, Washington State Association of Local Public Health Officials

Consulting (non voting)

Katrina & John Galipeau, parents
Sara Patton, parent
Kathy & Paul Riemann, parents
Peggy Harris, Save Babies Through Screening organization
Tara Wolff, State Board of Health
Maria Nardella, Dept. of Health, Children with Special Health Care Needs
Ala Mofidi, PhD, MBA, Dept. of Health, Policy, Legislative and Constituent Relations
Michael Glass, MS, Dept. of Health, Newborn Screening
John Thompson, MPH, MPA, Dept. of Health, Newborn Screening

CF Technical Advisory Committee Members

Cystic Fibrosis Medical/Clinical

1. Margaret Rosenfeld, MD, MPH, Children's Hospital CF clinic
2. Larry Larson, DO, Mary Bridge CF clinic
3. Susan Casey, RD, CD, Children's Hospital CF clinic

Public Health

4. Maxine Hayes, MD, MPH, State Health Officer, Washington State Dept. of Health
5. Tom Locke, MD, MPH, Chair, Washington State Board of Health
6. Tara Wolff, MPH, Health Policy Advisor, State Board of Health
7. Michael Glass, MS, Director Newborn Screening, Washington State Dept. of Health